



4160-01-P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2012-D-1038]

Guidance for Industry: Preclinical Assessment of Investigational Cellular and Gene Therapy Products; Availability

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice.

SUMMARY: The Food and Drug Administration (FDA) is announcing the availability of a document entitled "Guidance for Industry: Preclinical Assessment of Investigational Cellular and Gene Therapy Products" dated November 2013. The guidance document provides sponsors and individuals that design and implement preclinical studies with recommendations on the substance and scope of preclinical information needed to support clinical trials for investigational products reviewed by the Office of Cellular, Tissue and Gene Therapies (OCTGT). The product areas covered by this guidance are cellular therapy, gene therapy, therapeutic vaccination, xenotransplantation, and certain biologic-device combination products, which OCTGT reviews. The guidance clarifies current expectations regarding the preclinical information that would support an investigational new drug application (IND) and a biologics license application (BLA) for these products. The guidance announced in this notice finalizes the draft guidance of the same title dated November 2012, and supersedes the recommendations in Section VIII in the guidance entitled "Guidance for Industry: Guidance for Human Somatic Cell Therapy and Gene Therapy" dated March 1998.

DATES: Submit either electronic or written comments on Agency guidances at any time.

ADDRESSES: Submit written requests for single copies of the guidance to the Office of Communication, Outreach and Development (HFM-40), Center for Biologics Evaluation and Research (CBER), Food and Drug Administration, 1401 Rockville Pike, suite 200N, Rockville, MD 20852-1448. Send one self-addressed adhesive label to assist the office in processing your requests. The guidance may also be obtained by mail by calling CBER at 1-800-835-4709 or 301-827-1800. See the SUPPLEMENTARY INFORMATION section for electronic access to the guidance document.

Submit electronic comments on the guidance to <http://www.regulations.gov>. Submit written comments to the Division of Dockets Management (HFA-305), Food and Drug Administration, 5630 Fishers Lane, rm. 1061, Rockville, MD 20852.

FOR FURTHER INFORMATION CONTACT: Tami Belouin, Center for Biologics Evaluation and Research (HFM-17), Food and Drug Administration, 1401 Rockville Pike, suite 200N, Rockville, MD 20852-1448, 301-827-6210.

SUPPLEMENTARY INFORMATION:

I. Background

FDA is announcing the availability of a document entitled "Guidance for Industry: Preclinical Assessment of Investigational Cellular and Gene Therapy Products" dated November 2013. The guidance document provides sponsors and individuals that design and implement preclinical studies with recommendations on the substance and scope of preclinical information needed to support clinical trials for investigational products reviewed by OCTGT. The product areas covered by this guidance include cellular therapy, gene therapy, therapeutic vaccination, xenotransplantation, and certain biologic-device combination products. The guidance is

intended to clarify current expectations regarding the preclinical information that supports an IND and a BLA for these products.

In the Federal Register of November 29, 2012 (77 FR 71194), FDA announced the availability of the draft guidance of the same title dated November 2012. FDA received numerous comments on the draft guidance and those comments were considered as the guidance was finalized. In response to these comments, several sections were reorganized and editorial changes throughout the document were made to improve clarity. The guidance announced in this notice finalizes the draft guidance dated November 2012.

The guidance is being issued consistent with FDA's good guidance practices regulation (21 CFR 10.115). The guidance represents FDA's current thinking on this topic. It does not create or confer any rights for or on any person and does not operate to bind FDA or the public. An alternative approach may be used if such approach satisfies the requirement of the applicable statutes and regulations.

II. Paperwork Reduction Act of 1995

This guidance refers to previously approved collections of information found in FDA regulations. These collections of information are subject to review by the Office of Management and Budget (OMB) under the Paperwork Reduction Act of 1995 (44 U.S.C. 3501-3520). The collections of information in 21 CFR part 312 have been approved under OMB control number 0910-0014; the collections of information in 21 CFR part 601 have been approved under OMB control number 0910-0338; the collections of information in 21 CFR part 58 have been approved under OMB control number 0910-0119.

III. Comments

Interested persons may submit electronic comments regarding this document to <http://www.regulations.gov> or written comments to the Division of Dockets Management (see ADDRESSES). It is only necessary to send one set of comments. Identify comments with the docket number found in brackets in the heading of this document. Received comments may be seen in the Division of Dockets Management between 9 a.m. and 4 p.m., Monday through Friday, and will be posted to the docket at <http://www.regulations.gov>.

IV. Electronic Access

Persons with access to the Internet may obtain the guidance at either <http://www.fda.gov/BiologicsBloodVaccines/GuidanceComplianceRegulatoryInformation/Guidances/default.htm> or <http://www.regulations.gov>.

Dated: November 19, 2013.

Leslie Kux,
Assistant Commissioner for Policy.

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